Title (en)

IMPROVED RAAV VECTORS AND METHODS FOR TRANSDUCTION OF PHOTORECEPTORS AND RPE CELLS

Title (de)

VERBESSERTE RAAV-VEKTOREN UND VERFAHREN ZUR TRANSDUKTION VON FOTOREZEPTOREN UND RPE-ZELLEN

Title (fr)

VECTEURS RAAV AMÉLIORÉS ET PROCÉDÉS POUR LA TRANSDUCTION DE PHOTORÉCEPTEURS ET CELLULES EPR

Publication

EP 3113787 A1 20170111 (EN)

Application

EP 15758026 A 20150304

Priority

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- US 2015018791 W 20150304

Abstract (en)

[origin: WO2015134643A1] Disclosed are capsid-modified rAAV particles and expression vectors, as well as compositions and pharmaceutical formulations that comprise them. Also disclosed are methods of preparing and using novel capsid-protein-mutated particle or rAAV vector constructs in a variety of diagnostic and therapeutic applications including, inter alia, as delivery agents for diagnosis, treatment, or amelioration of one or more diseases, disorders, or dysfunctions of the mammalian eye. Also disclosed are methods for subretinal delivery of therapeutic gene constructs to mammalian photoreceptors and retinal pigment epithelial cells, as well as use of the disclosed compositions in the manufacture of medicaments for a variety of in vitro and/or in vivo applications including the treatment of a variety of inherited retinal diseases.

IPC 8 full level

A61K 38/16 (2006.01)

CPC (source: EP US)

A61K 9/0048 (2013.01 - EP US); **A61K 9/5184** (2013.01 - EP US); **C07K 14/005** (2013.01 - EP US); **C12N 15/86** (2013.01 - US); A61K 48/00 (2013.01 - US); C12N 2750/14122 (2013.01 - EP US); C12N 2750/14143 (2013.01 - EP US); C12N 2810/6027 (2013.01 - EP US)

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DOCDB simple family (publication)

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